HEALTH TECHNOLOGY ASSESSMENT FROM AN EU PERSPECTIVE*

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ABA Section of International Law
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* With thanks to Koosje van Lessen Kloeke (Leijnse Artz) who drafted a part of this slide deck

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Key themes international discussions

- Ensuring patients' access to medicines
- Lack of price transparency, access to medicines endangered by very high and unsustainable price levels
- Bottom-up approach, commonly felt need to address the situation jointly
- Potential areas for voluntary structured cooperation, i.a.:
  - Joint horizon scanning
  - Information-sharing
  - **Health Technology Assessment (HTA) cooperation**
  - Voluntary price negotiations

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What is **HTA?**

- Systematic evaluation of properties, effects and/or impacts of health technology; multidisciplinary process to evaluate the social, economic, organizational and ethical issues of a health intervention or health technology

- **Two aspects** (but can be limited to one):

  - **clinical assessment**:
    How well does a new technology work compared with existing alternative health technologies? For which population group does it work best? etc.

  - **economic assessment:**
    What costs are entailed for the health system?

- Main purpose: to inform policy decision making, ensuring accessibility, quality and sustainability of healthcare

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- EU: Directive 2011/24/EU – application of patients' rights in cross-border healthcare, i.a. cooperation on HTA (**EUnetHTA**):
  - create effective and sustainable network for HTA across Europe
  - develop reliable, timely, transparent and transferable information to contribute to HTA's in European countries
  - support collaboration between European HTA organizations that brings value at the European, national and regional level through:
    - facilitating efficient use of resources available for HTA
    - creating a sustainable system of HTA knowledge sharing
    - promoting good practice in HTA methods and processes

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eunethta
EUROPEAN NETWORK FOR HEALTH TECHNOLOGY ASSESSMENT

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HTA Core Model®

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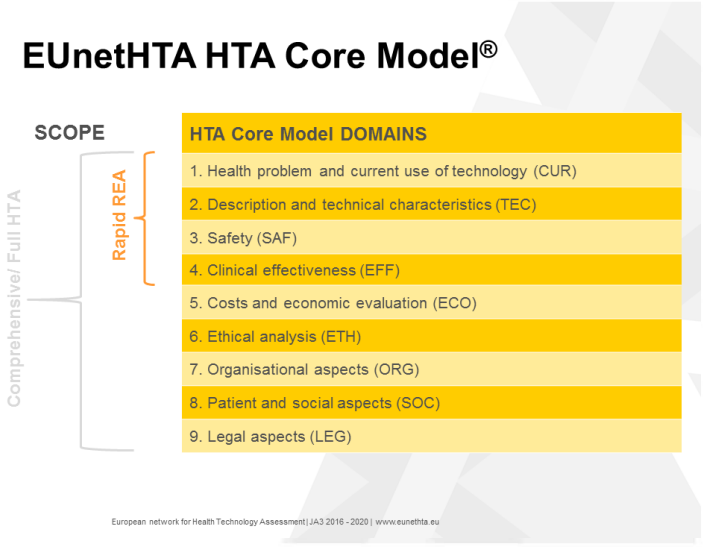
The HTA Core Model® is a *methodological framework* for production and sharing of HTA information. The HTA Core Model® is a registered trademark.



The model consists of the following three components, each with a specific purpose:

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PROPOSAL FOR A REGULATION

EU cooperation on Health Technology Assessment

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About this initiative

Reference	COM(2018)51
Type	Proposal for a regulation
Full title	Proposal for a REGULATION OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL on health technology assessment and amending Directive 2011/24/EU
Department	Directorate-General for Health and Food Safety

Proposal for a regulation - COM(2018) 51/DOCUMENT-2018-3552

English (533 KB - PDF - 49 pages)

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EU Commission Proposal on Joint HTA (I)

- Smaller Member States more in favour (fewer resources) but concerns about higher prices and lowering of standards
- Major issues for Member States
  - Autonomy and independence of HTA agencies:
    - Autonomy: *mandatory* use of joint clinical assessment report
    - Independence: approval of joint assessments before publication
- Availability of all evidence
- Capacity of HTA agencies

EU Commission Proposal on Joint HTA (II)

› Autonomy and independence of HTA agencies:

› Autonomy: mandatory use of joint clinical assessment report

› Proposal states that

> *"the joint clinical assessments will be one of the main proponents of the future joint work and, following the end of the transitional period, participation in the assessments and use of the joint clinical assessment reports at Member State-level will be mandatory" and that "Where Member States do carry out HTAs on such health technologies, there is a requirement for mandatory use of the joint clinical assessment report and no repetition of the clinical assessment in Member States' overall HTA processes"*
>
> (emphasis added)

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EU Commission Proposal on Joint HTA (III)

› A critical assessment of the joint clinical assessment and adaptation to the national context should always remain possible

› HTA bodies should be able to adapt joint clinical assessment to national situation (*e.g.*, selection of appropriate comparator, use of national administrative databases, etc.)

› Update of evidence by individual HTA body should remain possible

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EU Commission Proposal on Joint HTA (IV)

› Autonomy and independence of HTA agencies:

› Independence: approval of joint assessments before publication

> *"The proposal would place on the **Commission an obligation to verify the joint clinical assessment reports prior to their publication**" and "The Commission shall publish the approved joint clinical assessment report and summary report on the IT platform"* (emphasis added)

› HTA bodies should be able to publish their independent assessments without interference of another body

› Commission should not have possibility to block publication of assessment

› Authors of report should have the opportunity to respond to commission's comments

› Researchers performing an assessment should be allowed to publish their findings on website of their HTA institute and/or in peer-reviewed journals free from outside interference

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EU Commission Proposal on Joint HTA (V)

› Availability of all evidence

Proposal states that "*the designated sub-group shall* ***request the health technology developer to submit*** *the documentation containing the information,* ***data and evidence*** *necessary for the joint scientific consultation"* (emphasis added)

› HTA experts are confronted with a major problem of publication and reporting bias.

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EU Commission Proposal on Joint HTA (VI)

› The proposal includes insufficient obligations for the technology developers to provide all evidence:

1.
Timely prospective registration of all trials

This should allow assessors to check whether all evidence has been submitted. The timely registration should be monitored and necessary steps should be taken if the technology developer fails to comply.

2.
Provide a full list of all studies

A list should be provided of all studies in which the technology has been used. The status of these studies should be provided (ongoing, stopped, finished, etc.). The results or reasons for stopping the study should be provided.

3.
Information should be provided in a transparent and structured manner

The technology developers should submit their data according to a standardised template (e.g. ordered per study type, proper summary tables (e.g. on adverse events), access to underlying data to be able to check the information in the file, etc.).

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EU Commission Proposal on Joint HTA (VII)

› Capacity of HTA agencies

› Proposal states that

> *Participation in the assessments and use of the joint clinical assessment reports at Member State level will be mandatory", that "The designated authorities and bodies should ensure an appropriately high level of representation in the Coordination Group and technical expertise in its sub-groups, taking into account the need to provide expertise on the HTA of medicinal products and medical devices", that "Following the end of the transitional period, all medicinal products falling within the scope and granted marketing authorisation in a given year will be assessed, while a selection of medical devices falling within the scope will undergo assessment" and that "Members States which are already participating should not be allowed to withdraw from the framework for joint work"*

› It is very questionable that all HTA agencies have sufficient capacity to perform their work for both their own government as well as the work for all medicinal products falling within the scope.

EU Commission Proposal on Joint HTA (VIII)

- Draft Report of 4 May 2018 of EP Committee on the Environment, Public Health and Food Safety (available here)
- Next step: Health Council meeting of 22 June 2018 in Luxembourg to discuss the draft Regulation
- Aim of Austria - Council presidency in second half of 2018: new text on EU-level HTA by the end of 2018. Ease concerns regarding Commission's proposed mandatory uptake of joint clinical assessments
- Latest discussions suggest that compromise is possible: Germany "*is not intending to kill any proposal*" and Italian 5Star Movement also appears to support EU-level HTA

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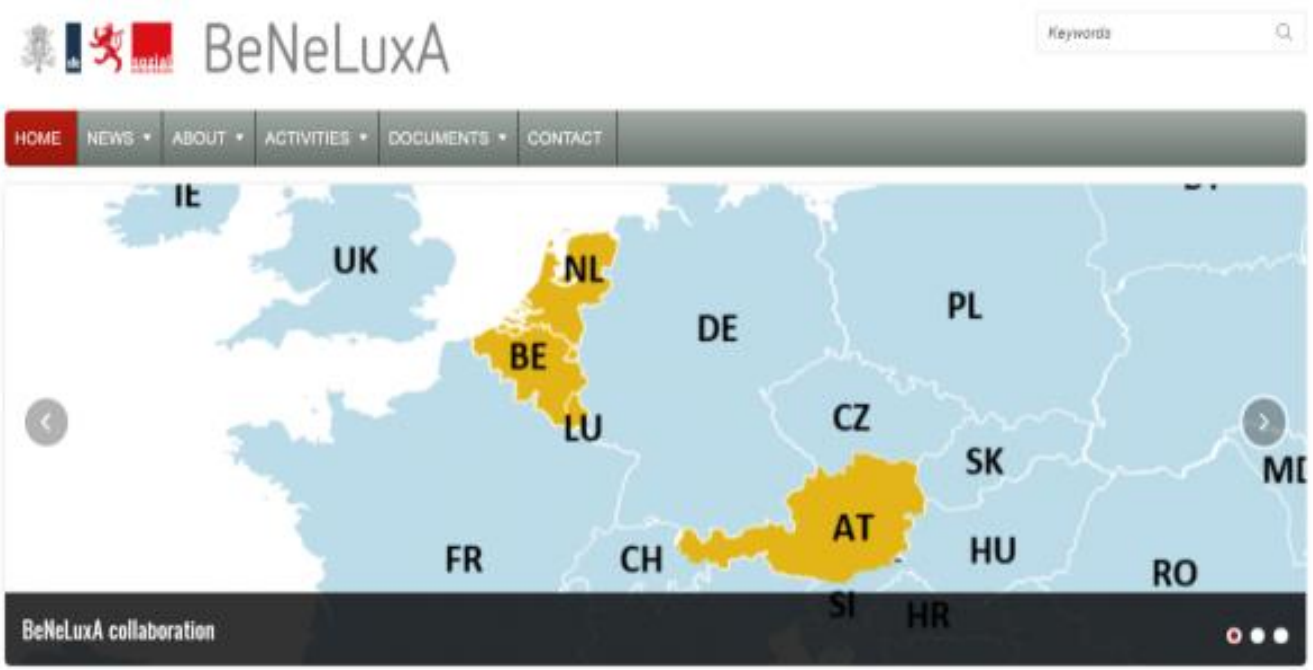
BeNeLuxA



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Participants

› Initiated by Belgium and the Netherlands (April 2015)

› Later joined by Luxembourg (September 2015) and Austria (June 2016)

› Ireland will join on 22 June 2018

› Population covered by initiative is 38 million inhabitants (incl. Ireland: 43 million)

› Operational coordination for BeNeLuxA rotates, currently The Netherlands

› *Note: Other initiatives exist, e.g. Valetta Declaration group, Visegrad group and Nordics cooperation, but at present are less advanced. Ireland is also a member of the Valetta Declaration Group.*

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Future participants

- BeNeLuxA is willing to consider including other countries (including non-EU) into their proposal
- Cooperation is not necessarily limited to EU Member States
- **Switzerland** is interested in joining the initiative
- **France** is interested in collaborating with the group on (1) Exchange of information; (2) Horizon Scanning; and (3) HTA
- **Italy** has also approached the Netherlands to explore the possibilities for further collaboration (informally)

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Transparency?

› Work in progress ("*learning by doing*")

› Limited information available on nature, scope, criteria and practical details of the BeNeLuxA Initiative

› Scattered information

› Government-run website with public information re. initiative: www.beneluxa.org

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Legal context

› Constitutional systems countries

› EU law (i.a. free movement of goods, Transparency Directive, Directive 2011/24/EU)

› BeNeLuxA is not based on a treaty

› Based on letters of intent and additional working agreements for administrative consultations, i.a. joint horizon scanning agreement (BeNeLuxA – Terms of Reference)

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Scope

› ‘BeNeLuxA’ intends to collaborate more closely across a range of areas

› Initiative goes beyond jointly negotiating with the pharmaceutical industry

› Aim of the initiative is twofold:

a. Increasing clout by sharing knowledge and expertise

b. Increasing market power by having joint negotiations

› Non-exclusive

› No duplication of work in other fora (e.g. EUnetHTA)

› Connection with other joint initiatives, other countries is possible

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Framework joint assessment

› Still a pilot phase

› Scope: not limited to orphan medicines; open to any medicine with a significant budgetary or therapeutic impact. Currently no plans to include medical devices

› Includes both clinical and economic assessment (compare Commission proposal: only clinical assessment)

› Framework for joint HTA assessment still under development at BeNeLuxA level

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Four types of HTA collaboration

Re-use of HTA reports

- Countries use parts of HTA-reports of other countries

Joint HTA report

- Authors of several countries join forces in order to write one report, which can then be used in all the countries involved

Mutual recognition

- HTA-report of one country (in part or full report) is adopted by others in a parallel process; the results of the assessments are then published at the same time

External referee

- HTA institutes of the various countries act as an external referee for another country in national procedures. It does not involve active work in HTA itself

First results of joint HTA procedures (October 2017)

Name pharmaceutical	active substance (EMA)	therapeutic area (EMA)	year	Type of HTA-collaboration
LOJUXTA	lomitapide	hyper-cholesterolemia	2015	Re-use of Dutch work by Belgium
ORKAMBI	lumacaftor / ivacaftor	cystic fibrosis	2016 first submission	Joint writing by Belgium & The Netherlands The Dutch Zorginstituut also acted as external referee for RIZIV-INAMI Final report was used by Luxembourg
PRALUENT	alirocumab	dyslipidemias	2016	Dutch Zorginstituut acted as external referee for Belgium RIZIV-INAMI
ORKAMBI	lumacaftor / ivacaftor	cystic fibrosis	2017 second submission	Joint writing by Belgium & The Netherlands The Dutch Zorginstituut also acted as external referee for RIZIV-INAMI Final report was sent to Luxembourg and Austria
VYNDAQEL	tafamidis	amyloidosis	2017	The Dutch Zorginstituut acted as external referee for RIZIV-INAMI Final report was used by Luxembourg

The Table mentions the situation in October 2017.

Abbreviations: RIZIV-INAMI Rijksinstituut voor Ziekte- en Invaliditeitsverzekering Institut National Assurance Maladie-Invalidité(Belgian HTA activities on submitted pharmaceutical files for reimbursement); EMA European Medicines Agency

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Main challenges (I)

› Identification of differences in national legal systems and procedures

› Starting points for the pharmaco-therapeutic assessment have to be similar in the countries, any differences in national clinical practice, criteria, etc. have to be identified beforehand (*e.g.* different start criteria for treatment)

› Countries use different application templates

› Do the national rules allow consultations of experts in the field?

› Pharmaco-economic evaluation required for orphan drug?

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Main challenges (II)

› Language of communications: Dutch, French, German, English? (e.g. no legal requirement in the Netherlands to draft agreements in Dutch)

› National language requirements in HTA (e.g. Belgium and Austria: not possible to submit HTA application in English and/or to draft authorities' assessment report in English; etc.?

› Governing law and jurisdiction?

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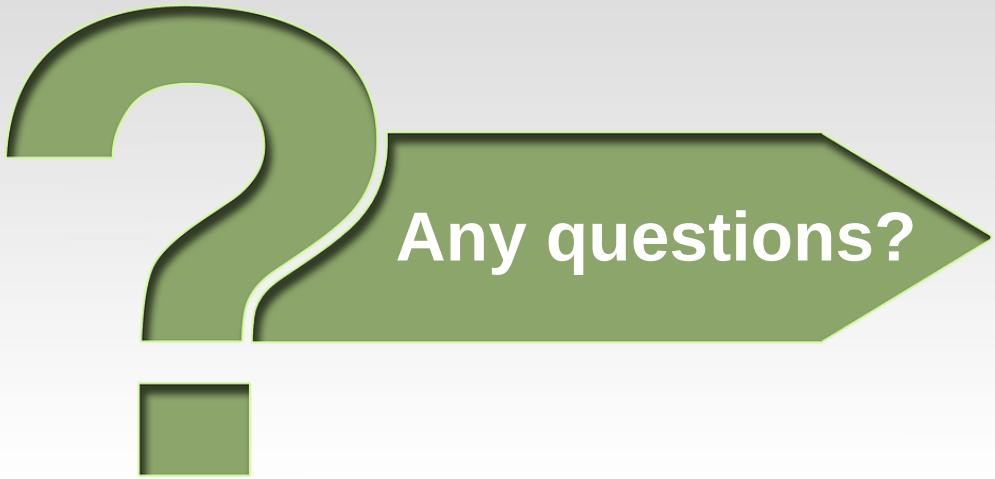

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